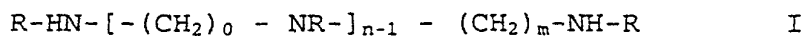


CLAIMS

1. Lipid compound of formula:



in which:

the R residues are, independently of each other, a hydrogen atom or a group of formula II:



for which:

R<sub>1</sub> and R<sub>2</sub> are, independently of each other, C<sub>6</sub>-C<sub>23</sub> alkyl or alkenyl radicals, which are linear or branched, or radicals -C(=O)-(C<sub>6</sub>-C<sub>23</sub>) alkyl or -C(=O)-(C<sub>6</sub>-C<sub>23</sub>) alkenyl, which are linear or branched, aryl radicals, cycloalkyl radicals, fluoroalkyl radicals, polyethylene glycol groups, oxyethylene or oxymethylene groups which are optionally repeated, linear or branched, optionally substituted,

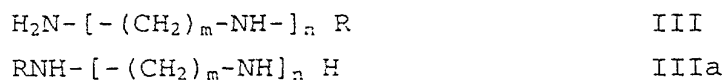
p is a positive integer from 1 to 4,

n is a positive integer from 1 to 6,

m is a positive integer from 1 to 6 which may be different for each motif -(CH<sub>2</sub>)<sub>m</sub>, and more particularly for each motif -(CH<sub>2</sub>)<sub>m</sub>-NR- when n > 1,

the number of R groups of formula II being between 1 and 4.

2. Compound according to claim 1, chosen from the compounds of formulae:



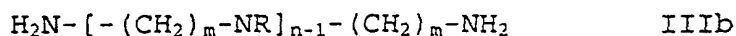
in which:

R is a group of formula II as defined in claim 1,

n is a positive integer from 1 to 6,

m is a positive integer from 1 to 6 which may be different for each motif  $-(CH_2)_m$ .

3. Compound according to claim 1 of formula:



10 in which:

R has one of the meanings indicated for the formula I of claim 1 provided that at least one R group is of formula II,

n is a positive integer from 1 to 6,

15 m is a positive integer from 1 to 6 which may be different for each motif  $-(CH_2)_m$ , and more particularly for each motif  $-(CH_2)_m-NR-$  when  $n > 1$ .

4. Compound according to claim 3, characterized in that it contains one or two R groups of formula II.

20 5. Compound according to claims 1 to 4, characterized in that  $R_1$  and  $R_2$  are, independently of each other, linear  $-C(=O)$ -alkyl or linear  $-C(=O)$ -alkenyl radicals.

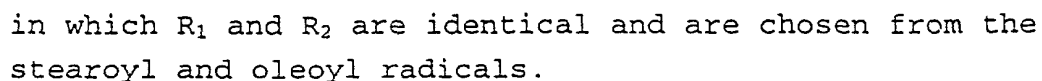
6. Compound according to claim 5, characterized in that said alkyl or alkenyl comprises from 12 to 20 carbon atoms and in that said compound comprises 1 or 2 R groups of formula II.

7. Compound according to claim 6, characterized in that said alkyl or alkenyl comprises 12, 16 or 18 carbon atoms.

8. Compound according to claims 1 to 7, characterized in that n is an integer chosen from the numbers 2, 3 or 4.

9. Compound according to claims 1 to 8, 35 characterized in that m is an integer chosen from the numbers 2, 3 or 4.

10. Compound according to claim 1, characterized in that it is chosen from the group consisting of the compounds of the following formulae:



11. Compound according to one of claims 1 to 10,  
25 characterized in that it is conjugated with one or more  
ligands of interest via one of the secondary or primary  
nitrogen atoms of the polyamine chain or of the  
diaminocarboxylic acid.

12. Compound according to claim 11, characterized  
30 in that said targeting component is chosen from the  
group consisting of all or part of sugars, peptides,  
oligonucleotides, lipids, hormones, vitamins, antigens,  
antibodies, ligands specific for membrane receptors,  
ligands capable of reacting with an anti-ligand,  
35 fusogenic peptides, nuclear localization peptides, or a  
combination of such compounds.

13. Compound according to one of claims 1 to 12, characterized in that it is in a cationic form.

14. Composition characterized in that it comprises at least one compound according to one of the preceding claims and optionally at least one adjuvant capable in particular of enhancing the formation of the complex  
5 between said compound and an active substance.
15. Composition according to claim 14, characterized in that said adjuvant is a neutral or zwitterionic lipid.
16. Composition according to claim 15,  
10 characterized in that said neutral or zwitterionic lipid is or is derived from a triglyceride, a diglyceride, cholesterol, a phosphatidylethanolamine (PE), phosphatidylcholine, phosphocholine, sphyngo-myelin, ceramide or cerebroside.
- 15 17. Composition according to claim 16, characterized in that said neutral or zwitterionic lipid is dioleylephosphatidylethanolamine (DOPE).
18. Composition according to claims 14 to 17, characterized in that the compound/adjuvant weight  
20 ratio is between 0.1 and 10.
19. Complex comprising at least one compound according to claim 13 or at least one composition according to one of claims 14 to 18 and at least one active substance, in particular a therapeutically  
25 active substance, comprising at least one negative charge.
20. Complex according to claim 19, characterized in that said active substance is chosen from nucleic acids and proteins.
- 30 21. Complex according to claim 20, characterized in that said active substance is a nucleic acid chosen from the group consisting of a cDNA, a genomic DNA, a plasmid DNA, an antisense polynucleotide, a messenger RNA, a ribosomal RNA, a ribozyme, a transfer RNA, or a  
35 DNA encoding such RNAs.
22. Complex according to claim 21, characterized in that said nucleic acid comprises a gene of interest and components allowing the expression of said gene of interest.

23. Complex according to one of claims 19 to 22, characterized in that it has a size of less than 500 nm, advantageously less than 200 nm.

24. Complex according to one of claims 23,  
5 characterized in that it has a size of less than 100 nm.

25. Complex according to one of claims 19 to 24, characterized in that the ratio between the number of positive charges of the cationic compound(s) and/or  
10 composition(s) and the number of negative charges of said active substance varies from 0.05 to 20, more particularly from 0.1 to 15, and preferably from 5 to 10.

26. Process for preparing a complex according to  
15 claims 19 to 25, characterized in that one or more compounds according to claim 13 and/or at least one composition according to one of claims 14 to 18 are brought into contact with one or more active substances comprising at least one negative charge and in that  
20 said complex is recovered, optionally after a purification step.

27. Process of preparation according to claim 26, characterized in that said compounds and/or compositions are dissolved beforehand in a solvent  
25 which is miscible with water, in particular ethanol or dimethyl sulfoxide or a mixture of both.

28. Process of preparation according to claim 26, characterized in that said compounds and/or compositions are suspended beforehand in a detergent  
30 solution.

29. Process of preparation according to claim 28, characterized in that, in addition, a step of purification of said complex by dialysis is carried out.

35 30. Use of a compound according to any one of claims 1 to 13, of a composition according to any one of claims 14 to 18, of a complex according to any one of claims 19 to 25 to transfer at least one active substance, especially a therapeutically active

substance, in particular a nucleic acid, into target cells *in vitro*, *ex vivo* or *in vivo*, more particularly *in vivo*.

31. Use according to claim 30, characterized in that said target cell is a mammalian cell.

32. Use according to claim 31, characterized in that said target cell is selected from a muscle cell, a hematopoietic stem cell, a cell of the airways, more particularly a tracheal or pulmonary cell.

33. Process for transferring an active substance comprising at least one negative charge, into a cell, characterized in that said cell is brought into contact with a complex according to any one of claims 19 to 25.

34. Complex according to one of claims 19 to 25, as a medicament for curative, preventive or vaccinal purposes.

35. Complex according to one of claims 19 to 25 for carrying out a method of therapeutic treatment which consists in transferring at least one therapeutically active substance, in particular a nucleic acid, into target cells.

36. Complex according to claim 35, characterized in that said target cell is a mammalian cell.

37. Complex according to claim 36, characterized in that said target cell is selected from a muscle cell, a hematopoietic stem cell, a cell of the airways, more particularly a tracheal or pulmonary cell, a cell of the respiratory epithelium.

38. Use of a compound according to any one of claims 1 to 13, of a composition according to any one of claims 14 to 18, of a complex according to any one of claims 19 to 25 for the preparation of a medicament for curative, preventive or vaccinal purposes, intended for the treatment of the human or animal body, in particular by gene therapy.

39. Use according to claim 38, characterized in that the medicament is intended to be administered by intramuscular injection, by inhalation, by intratracheal injection, by instillation, by

aerosolization, by the topical route or by the oral route.

40. Pharmaceutical preparation characterized in that it comprises at least one complex according to any  
5 one of claims 19 to 25.

41. Preparation according to claim 40, characterized in that it comprises, in addition, at least one adjuvant capable of enhancing the transfecting power of said complex.

10 42. Preparation according to claim 41, characterized in that said adjuvant is chosen from the group consisting of chloroquine, a protic polar compound chosen in particular from propylene glycol, polyethylene glycol, glycerol, ethanol, 1-methyl-L-2-  
15 pyrrolidone or derivatives thereof, or an aprotic polar compound chosen in particular from dimethyl sulfoxide (DMSO), diethyl sulfoxide, di-n-propyl sulfoxide, dimethyl sulfone, sulfolane, dimethylformamide, dimethylacetamide, tetramethylurea, acetonitrile or  
20 derivatives thereof.

43. Preparation according to one of claims 40 to 42, characterized in that it comprises, in addition, a pharmaceutically acceptable carrier allowing its administration to humans or animals.

25 44. Cell transfected with a complex according to any one of claims 19 to 25.